



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2013-D-0446]

Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers; Draft Guidance for Industry; Availability

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of availability.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the availability of a revised draft guidance for industry entitled “Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers.” Since 2017, FDA has received many questions concerning implementation of the regulatory requirements of the expanded access program. In addition, FDA developed recommendations for fulfilling the new requirements for expanded access submissions promulgated in the 21st Century Cures Act (Cures Act) (2016) and the FDA Reauthorization Act of 2017 (FDARA). FDA is providing this guidance in a question-and-answer format, addressing the most recent frequently asked questions and sharing recommendations to fulfill the new statutory requirements. This guidance revises the guidance of the same title issued in June 2016 and updated in October 2017.

DATES: Submit either electronic or written comments on the draft guidance by [INSERT DATE 60 DAYS AFTER DATE OF PUBLICATION IN THE *FEDERAL REGISTER*] to ensure that the Agency considers your comment on this draft guidance before it begins work on the final version of the guidance.

ADDRESSES: You may submit comments on any guidance at any time as follows:

Electronic Submissions

Submit electronic comments in the following way:

- Federal eRulemaking Portal: <https://www.regulations.gov>. Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to <https://www.regulations.gov> will be posted to the docket unchanged. Because your comment will be made public, you are solely responsible for ensuring that your comment does not include any confidential information that you or a third party may not wish to be posted, such as medical information, your or anyone else's Social Security number, or confidential business information, such as a manufacturing process. Please note that if you include your name, contact information, or other information that identifies you in the body of your comments, that information will be posted on <https://www.regulations.gov>.
- If you want to submit a comment with confidential information that you do not wish to be made available to the public, submit the comment as a written/paper submission and in the manner detailed (see "Written/Paper Submissions" and "Instructions").

Written/Paper Submissions

Submit written/paper submissions as follows:

- Mail/Hand delivery/Courier (for written/paper submissions): Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.
- For written/paper comments submitted to the Dockets Management Staff, FDA will post your comment, as well as any attachments, except for information submitted, marked and identified, as confidential, if submitted as detailed in "Instructions."

Instructions: All submissions received must include the Docket No. FDA-2013-D-0446 for "Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers." Received comments will be placed in the docket and, except for those submitted as "Confidential Submissions," publicly viewable at <https://www.regulations.gov> or at the Dockets Management Staff between 9 a.m. and 4 p.m., Monday through Friday, 240-402-7500.

- Confidential Submissions--To submit a comment with confidential information that you do not wish to be made publicly available, submit your comments only as a written/paper submission. You should submit two copies total. One copy will include the information you claim to be confidential with a heading or cover note that states “THIS DOCUMENT CONTAINS CONFIDENTIAL INFORMATION.” The Agency will review this copy, including the claimed confidential information, in its consideration of comments. The second copy, which will have the claimed confidential information redacted/blacked out, will be available for public viewing and posted on <https://www.regulations.gov>. Submit both copies to the Dockets Management Staff. If you do not wish your name and contact information to be made publicly available, you can provide this information on the cover sheet and not in the body of your comments and you must identify this information as “confidential.” Any information marked as “confidential” will not be disclosed except in accordance with 21 CFR 10.20 and other applicable disclosure law. For more information about FDA’s posting of comments to public dockets, see 80 FR 56469, September 18, 2015, or access the information at:
<https://www.govinfo.gov/content/pkg/FR-2015-09-18/pdf/2015-23389.pdf>.

Docket: For access to the docket to read background documents or the electronic and written/paper comments received, go to <https://www.regulations.gov> and insert the docket number, found in brackets in the heading of this document, into the “Search” box and follow the prompts and/or go to the Dockets Management Staff, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852, 240-402-7500.

You may submit comments on any guidance at any time (see 21 CFR 10.115(g)(5)).

Submit written requests for single copies of the draft guidance to the Division of Drug Information, Center for Drug Evaluation and Research, Food and Drug Administration, 10001 New Hampshire Ave., Hillandale Building, 4th Floor, Silver Spring, MD 20993-0002; or the Office of Communication, Outreach and Development, Center for Biologics Evaluation and

Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 3128, Silver Spring, MD 20993-0002. Send one self-addressed adhesive label to assist that office in processing your requests. See the SUPPLEMENTARY INFORMATION section for electronic access to the draft guidance document.

FOR FURTHER INFORMATION CONTACT: Dat Doan, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 51, Rm. 3334, Silver Spring, MD 20993-0002, 240-402-8926; or Stephen Ripley, Center for Biologics Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 71, Rm. 7301, Silver Spring, MD 20993-0002, 240-402-7911.

SUPPLEMENTARY INFORMATION:

I. Background

FDA is announcing the availability of a revised draft guidance for industry entitled “Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers.” Under the expanded access regulation provided in 21 CFR part 312, subpart I, FDA allows use of investigational drugs for treatment of patients with serious or immediately life-threatening diseases or conditions who lack therapeutic alternatives. FDA issued a guidance in 2016 (updated in 2017) in a question-and-answer format to respond to the most frequently asked questions on various provisions of the regulation regarding expanded access.

The Cures Act added section 561A to the Federal Food, Drug, and Cosmetic Act (FD&C Act) (21 U.S.C. 360bbb-0) to include new requirements regarding expanded access. Under section 561A of the FD&C Act, a manufacturer or distributor of one or more investigational drugs for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions is required to make its policy for evaluating and responding to expanded access requests (expanded access policy) readily available to the public, such as by posting the policy on a publicly available website. In addition, FDARA (Pub. L. 115-52) amended the FD&C Act to require that the expanded access policy for an investigational drug be posted by the earlier of (1) the first

initiation of a phase 2 or phase 3 study with respect to such investigational drug or (2) within 15 days after the drug receives a fast track, breakthrough, or regenerative advanced therapy designation.

This revised draft guidance includes responses to stakeholder questions received since publication of the updated final guidance in 2017 and includes the Agency's recommendations related to new requirements of the Cures Act and FDARA that are related to expanded access. This guidance revises the guidance for industry of the same title issued in June 2016 and updated in October 2017. Significant changes to the 2017 version of the guidance include additional recommendations related to institutional review board review, informed consent, and new requirements established by the Cures Act and FDARA.

This revised draft guidance is being issued consistent with FDA's good guidance practices regulation (21 CFR 10.115). The revised draft guidance, when finalized, will represent the current thinking of FDA on "Expanded Access to Investigational Drugs for Treatment Use: Questions and Answers." It does not establish any rights for any person and is not binding on FDA or the public. You can use an alternative approach if it satisfies the requirements of the applicable statutes and regulations.

II. Paperwork Reduction Act of 1995

While this guidance contains no collection of information, it does refer to previously approved FDA collections of information. Therefore, clearance by the Office of Management and Budget (OMB) under the Paperwork Reduction Act of 1995 (PRA) (44 U.S.C. 3501-3521) is not required for this guidance. The previously approved collections of information are subject to review by OMB under the PRA. The collections of information in 21 CFR part 312 have been approved under OMB control number 0910-0014; the collections of information in 21 CFR parts 50 and 56 have been approved under OMB control number 0910-0130; the collections of information under the Expanded Access regulations (21 CFR 312.300 through 312.320) have

been approved under OMB control number 0910-0814; and the collections of information in 42 CFR part 11 have been approved under OMB control number 0925-0586.

III. Electronic Access

Persons with access to the internet may obtain the revised draft guidance at <https://www.fda.gov/drugs/guidance-compliance-regulatory-information/guidances-drugs>, <https://www.fda.gov/vaccines-blood-biologics/guidance-compliance-regulatory-information-biologics/biologics-guidances>, <https://www.fda.gov/regulatory-information/search-fda-guidance-documents>, or <https://www.regulations.gov>.

Dated: October 27, 2022.

Lauren K. Roth,

Associate Commissioner for Policy.

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